



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2018-N-2455]

Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments; Draft Guidance for Industry, Food and Drug Administration Staff, and Other Stakeholders; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft guidance for industry entitled “Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments.” This guidance (Guidance 3) is the third in a series of four methodological patient-focused drug development (PFDD) guidance documents that describe how stakeholders (patients, researchers, medical product developers, and others) can collect and submit patient experience data and other relevant information from patients and caregivers to be used for medical product development and regulatory decision-making. When finalized, Guidance 3 will represent the current thinking of the Center for Drug Evaluation and Research, the Center for Biologics Evaluation and Research, and the Center for Devices and Radiological Health on this topic.

DATES: Submit either electronic or written comments on the draft guidance by [INSERT DATE 90 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*] to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESSES: You may submit comments on any guidance at any time as follows:

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2018-N-2455 for "Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments." Received comments will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at

<https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday, 240-402-7500.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states “THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION.” The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as “confidential.” Any information marked as “confidential” will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA’s posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at:

<https://www.govinfo.gov/content/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the “Search” box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852, 240-402-7500.

You may submit comments on any guidance at any time (see 21 CFR 10.115(g)(5)).

Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001

New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993-0002, or to the Office of Communication, Outreach and Development, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Shannon Cole, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, Rm. 6306, Silver Spring, MD 20993-0002, 301-796-9208, Shannon.Cole@fda.hhs.gov; or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911; or Office of Strategic Partnerships and Technology Innovation, Center for Devices and Radiological Health, cdrh-pro@fda.hhs.gov, 800-638-2041 or 301-796-7100.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled “Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments.” This guidance (Guidance 3) is the third in a series of four methodological PFDD guidance documents that describe how stakeholders (patients, researchers, medical product developers, and others) can collect and submit patient experience data and other relevant information from patients and caregivers to be used for medical product development and regulatory decision-making. For purposes of this guidance a “medical product” refers to a drug (as defined in section 201 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 321)) intended for human use, a device (as defined in section 201), or a biological product (as defined in section 351 of the Public Health Service Act (42 U.S.C. 262)).

This series of guidance documents is intended to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful input that can more consistently inform medical product development and regulatory decision-making. Guidance 3 discusses approaches to selecting, modifying, developing, and validating clinical outcome assessments to measure outcomes of importance to patients in clinical trials.

This draft guidance is being issued consistent with FDA’s good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the current thinking of FDA on “Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments.” It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

While this guidance contains no collection of information, it does refer to previously approved FDA collections of information. Therefore, clearance by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (PRA) (44 U.S.C. 3501-3521) is not required for this guidance. The previously approved collections of information are subject to review by OMB under the PRA. This guidance refers to collections of information from “individuals under treatment or clinical examination in connection with research,” which are not subject to review by OMB under 5 CFR 1320.3(h)(5). Respondents submit to FDA collections of information to support the medical product’s effectiveness and to support claims in approved medical labeling.

The collections of information in 21 CFR 314.50, 314.126, and 601.2 are submitted to FDA to support the medical product’s effectiveness and to support claims in approved medical product labeling. The collections of information have been approved under OMB control numbers 0910-0001 and 0910-0338. The collections of information in 21 CFR 312.23 regarding investigational new drug applications, including clinical trial design and study protocols, have

been approved under OMB control number 0910-0014. The collections of information in 21 CFR parts 50 and 56 regarding institutional review boards and the protection of human subjects have been approved under OMB control number 0910-0130. The collections of information in 21 CFR part 11 regarding electronic records and signatures have been approved under OMB control number 0910-0303. The collections of information described in FDA’s guidance entitled “Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products” (<https://www.fda.gov/media/109951/download>) have been approved under OMB control number 0910-0429.

III. Additional Information

Section 3002 of Title III, Subtitle A, of the 21st Century Cures Act (Pub. L. 114-255) directs FDA to develop patient-focused drug development guidance to address a number of areas, including under section 3002(c)(2): Methodological approaches that may be used to develop and identify what is important to patients with respect to burden of disease, burden of treatment, and the benefits and risks in the management of the patient’s disease.

In addition, FDA committed to meet certain performance goals under the sixth authorization of the Prescription Drug User Fee Act. These goal commitments were developed in consultation with patient and consumer advocates, healthcare professionals, and other public stakeholders, as part of negotiations with regulated industry. Section J.1 of the commitment letter, “Enhancing the Incorporation of the Patient’s Voice in Drug Development and Decision-Making” (<https://www.fda.gov/downloads/ForIndustry/UserFees/PrescriptionDrugUserFee/UCM511438.pdf>) outlines work, including the development of a series of guidance documents and associated public workshops to facilitate the advancement and use of systematic approaches to collect and use robust and meaningful patient and caregiver input that can more consistently inform drug development, and, as appropriate, regulatory decision-making.

IV. Electronic Access

Persons with access to the internet may obtain the draft guidance at

<https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs>,

[https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-](https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances)

[biologics/biologics-guidances](https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances), [https://www.fda.gov/regulatory-information/search-fda-guidance-](https://www.fda.gov/regulatory-information/search-fda-guidance-documents)

[documents](https://www.fda.gov/regulatory-information/search-fda-guidance-documents), or <https://www.regulations.gov>.

Dated: June 24, 2022.

Lauren K. Roth,

Associate Commissioner for Policy.

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